

# Orphan Drug Commercial Models: Sustaining the rare disease business

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## Abstracts

Choosing the right commercial model for orphan drug success

The societal and commercial ambitions of orphan drug developers determine which commercial model will best meet their aims. Profit, potential and patient benefit all play a part, so which model is best and what are the considerations which underpin that choice?

This report analyses current orphan drug commercial models, assesses their benefits and challenges, assesses how the market is developing and, with the help of expert input, examines how they benefit “pure play” orphan drug developers and big pharma.

Answering key questions:

Meeting expectations: What factors are vital for orphan drugs commercial teams to ensure that their business model works for all stakeholders?

Key components: What are the key components of current commercial models and how are these evolving?

Hybrid best? What can be learnt from the hybrid (or partnership) commercial strategy as exemplified by Sanofi and Genzyme? Is this the way of the future?

In house: Roche and Novartis are pursuing an integrated strategy - what is the challenge for big pharma in taking this path?

Small developer challenges: What research and market access challenges exist for

“pure play” orphan drugs companies and how are they overcoming them?

Assessing success? Profit or patient benefit - what factors are critical for assessing orphan drug success?

### Key Topics Explored

Big pharma could learn lessons from smaller orphan drug players in their consultative and inclusive attitude to patients

Size doesn't matter. The dynamics of the orphan drugs market mean that company size is not a critical factor for success. The market is characterised by a high number of smaller orphan drug companies who are focussing on a range of rare diseases with high unmet needs.

Competition in the rare disease space is now a fact and the market dynamic is changing dramatically. What are the potential negative impacts on the wider orphan drug sector?

The fully integrated commercial model allows access to the resources and reach of big pharma but competing priorities can dilute the attention given to rare diseases.

### Expert Contributors

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## Contents

### **1 EXECUTIVE SUMMARY**

### **2 RESEARCH OBJECTIVES AND METHODOLOGY**

### **3 CONTRIBUTORS**

### **4 CURRENT STRUCTURES**

#### 4.1 Key findings

#### 4.2 Orphan drugs market overview

##### 4.2.1 What are orphan drugs?

##### 4.2.2 The orphan drugs market is increasing in value

###### 4.2.2.1 There are a large number of orphan drugs being approved each year

###### 4.2.2.2 Some orphan drugs can generate significant revenue

###### 4.2.2.3 New products will continue to drive growth

#### 4.3 Companies involved in orphan drugs

### **5 ORPHAN DRUGS COMMERCIAL MODELS AND LEADING COMPANIES**

#### 5.1 Key findings

#### 5.2 There are different structures for orphan drugs companies

##### 5.2.1 A fully integrated model is favoured by larger pharmaceutical companies

##### 5.2.2 Partnership or partially integrated models are being increasingly used.

##### 5.2.3 Independent and focused on orphan drugs

###### 5.2.3.1 Shire: increasing its focus on rare diseases

###### 5.2.3.2 BioMarin: a blueprint for orphan drug success

### **6 STRATEGIES FOR SUCCESS**

#### 6.1 Key findings

#### 6.2 Past orphan drugs success has been largely down to the unmet need

##### 6.2.1 Product Acquisition removes the risk but can deliver returns

##### 6.2.2 A focused R&D strategy is essential for success

#### 6.3 Education and awareness is an important part of the commercialisation process

##### 6.3.1 Stakeholder engagement is fundamental for orphan drugs

###### 6.3.1.1 Patients are seen as the most important stakeholder by many

###### 6.3.1.2 Companies have a role to play in educating and supporting physicians

6.3.1.3 Determining the value proposition is an important element for commercial success

6.3.2 A successful model is not necessarily the one that delivers the highest revenues

6.3.2.1 Traditional commercial measures of success are used for orphan drugs

6.3.2.2 Patient outcomes are increasingly important for orphan drugs

## **7 KEY CHALLENGES FOR CURRENT ORPHAN DRUGS MODELS**

### **7.1 Key findings**

7.1.1 Pricing of orphan drugs is expected to become more challenging

7.1.2 The orphan drugs market place is becoming more competitive

7.1.3 There are emerging challenges in the approval processes

7.1.4 The definition of an orphan drug is being scrutinised

## **8 EVOLUTION OF COMMERCIAL MODELS**

### **8.1 Key findings**

8.1.1 A more focused model is perceived as being most effective

8.1.2 Patient engagement will continue to be critical for the orphan drugs model

8.1.3 Innovation will be key for future orphan drug success

8.1.4 Out-sourcing commercial activities can maintain focus and competitiveness

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